



# *Forum on Regenerative Medicine*

*Workshop on Navigating the Manufacturing Process and Assuring the Quality of Regenerative Medicine Therapies*

## *Session 3: DESIGNING TECHNOLOGIES TO MEET THE MANUFACTURING NEEDS OF NEW REGENERATIVE THERAPIES*

Confidential. Not to be copied, distributed, or reproduced without prior approval.

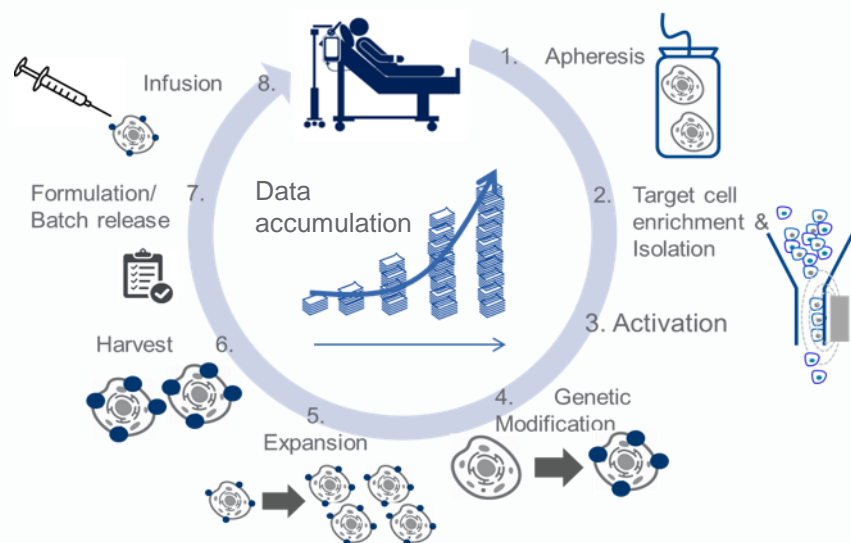
June 26, 2017

# Scaling Personalized Cell Therapy Manufacturing

## Current State

Cell therapy manufacturing today is:

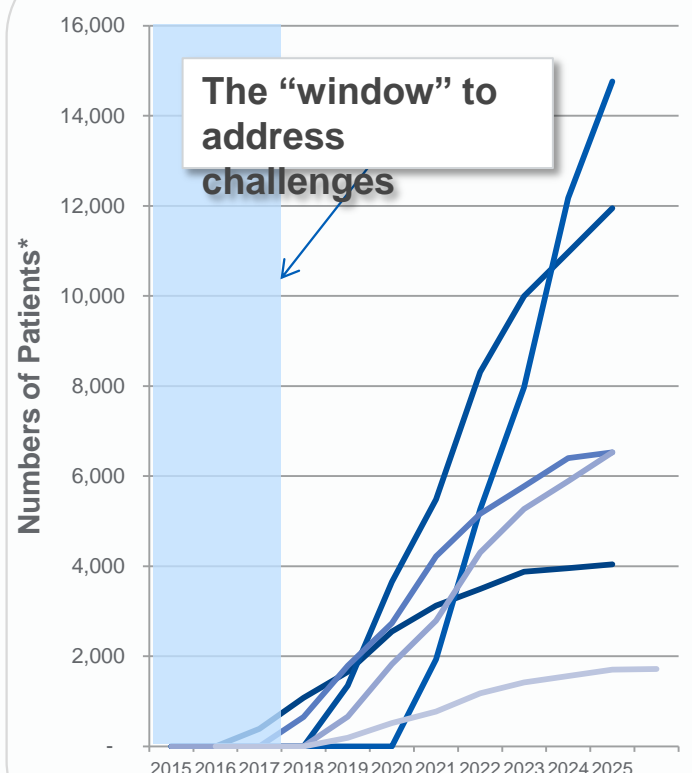
- Manual
- Complex
- Too many avoidable failures



- “Zero tolerance for failure” → “Every patient dose counts”

## Near-term State

### Predicted Scale-Out



\* CTT data on file, multiple indications shown

Required

~~Desired State~~

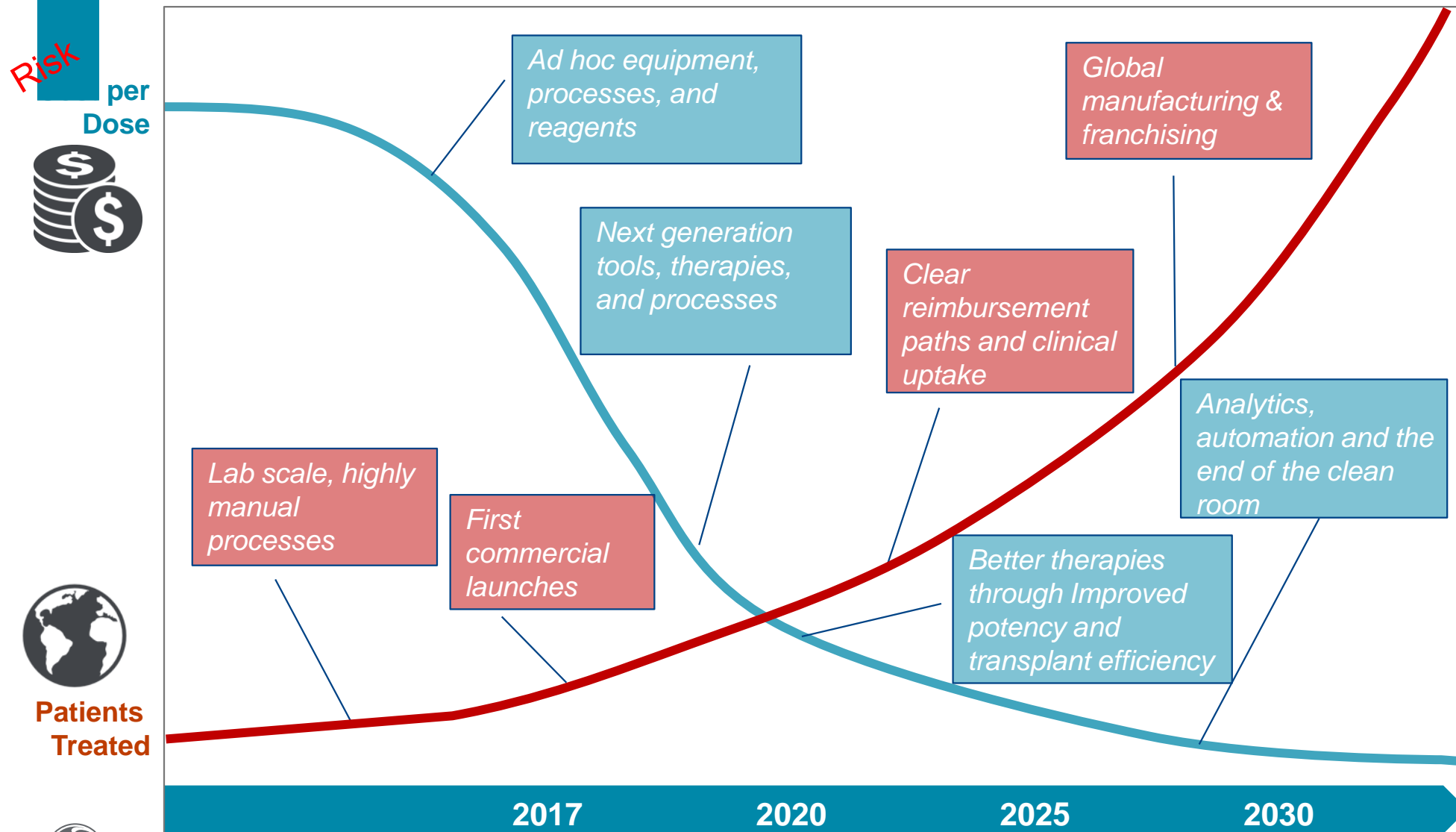
Cell Therapy Manufacturers want...

- ✓ operational excellence
- ✓ compliance
- ✓ a safe & effective

...cell therapy manufacturing solution to meet their scale-out needs

The ecosystem needs to evolve to treat patients “on demand”

# Predicting where the industry is going has huge impact on cell and gene therapy manufacturing

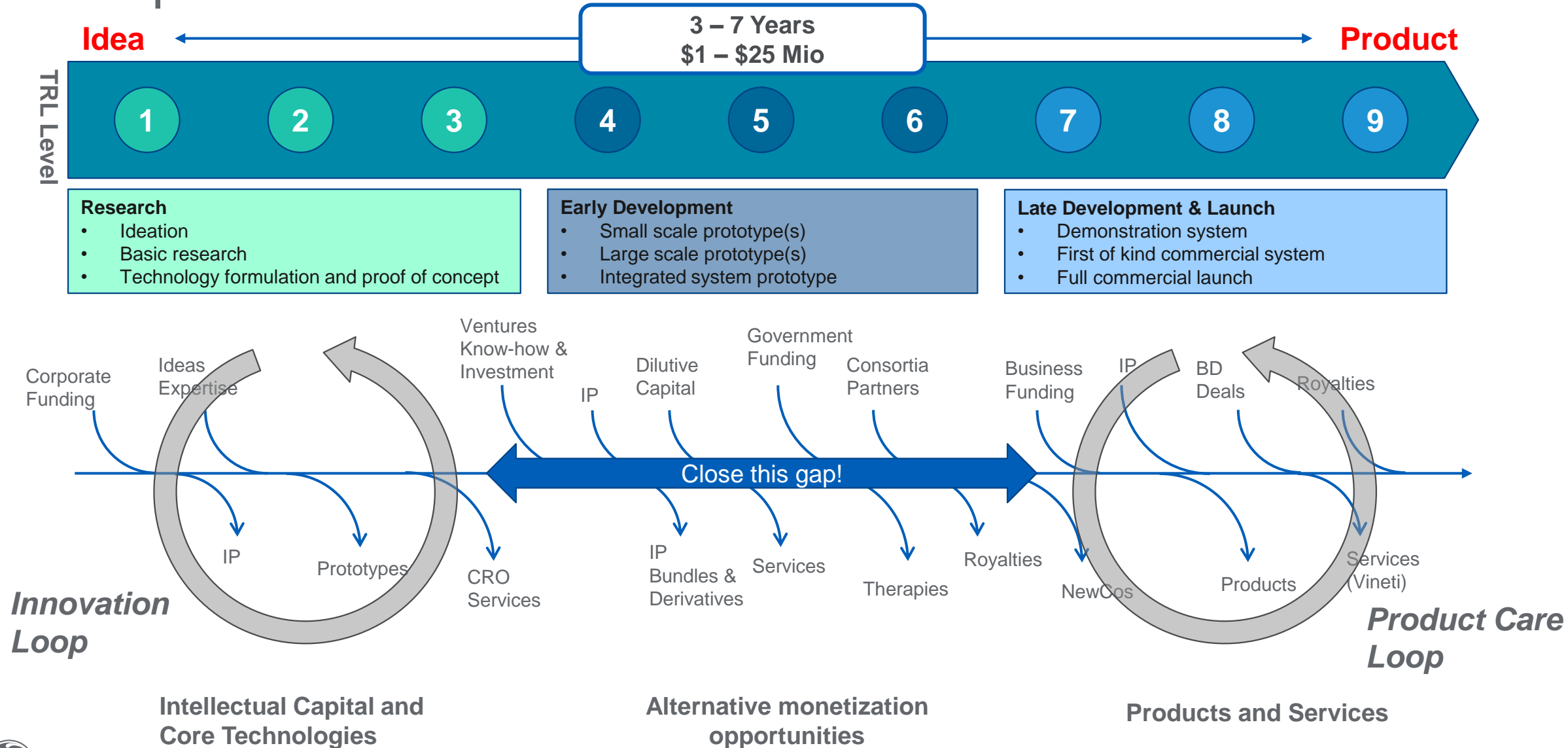


## Trend Summary

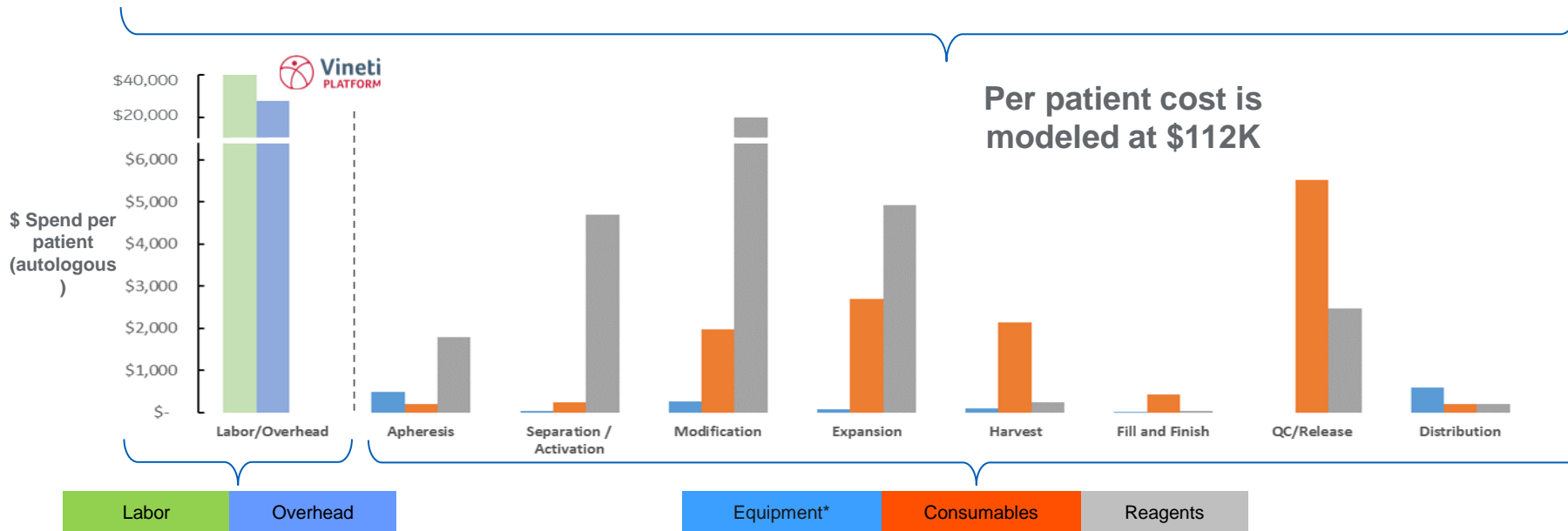
- 2-3 CAR-T market BLA's expected in 2017
- Insufficient global manufacturing capacity today
- Clinical/manufacturing /supply chain integration needed (Vitruvian)

- CAR-T COGS per dose greater than \$100K US
- Major cost driver - viral production adds \$10 to \$20K per dose
- Specialized labor intensity

# Challenge - It takes years and huge investment to bring new platforms to market



# Industrialization pain points are continually shifting



- Reagents and consumables are not produced at scale today, but will be
- Equipment capex is not a major driver at scale, but service and support will be increasingly critical
- Manufacturing capacity is not optimized
- Labor and lack of automation is major pain point still

- Equipment cost estimates based on a per patient basis. Revenue opportunity based on customer device demand is not fully represented
- \*\* Digital connectivity and Vineti are captured here



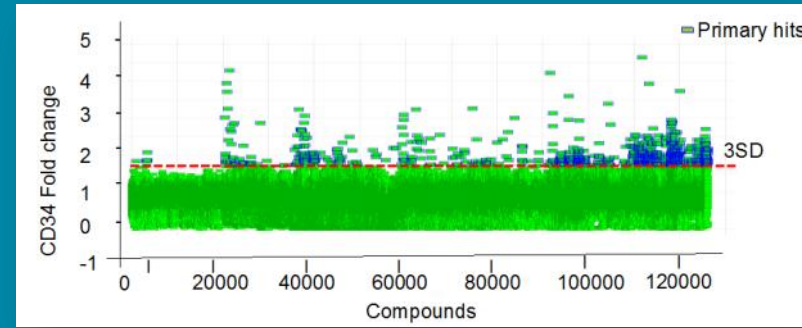
# Everything (almost) depends on dose size

Either, grow *more* cells cost effectively...



Convert to allogeneic universal cells

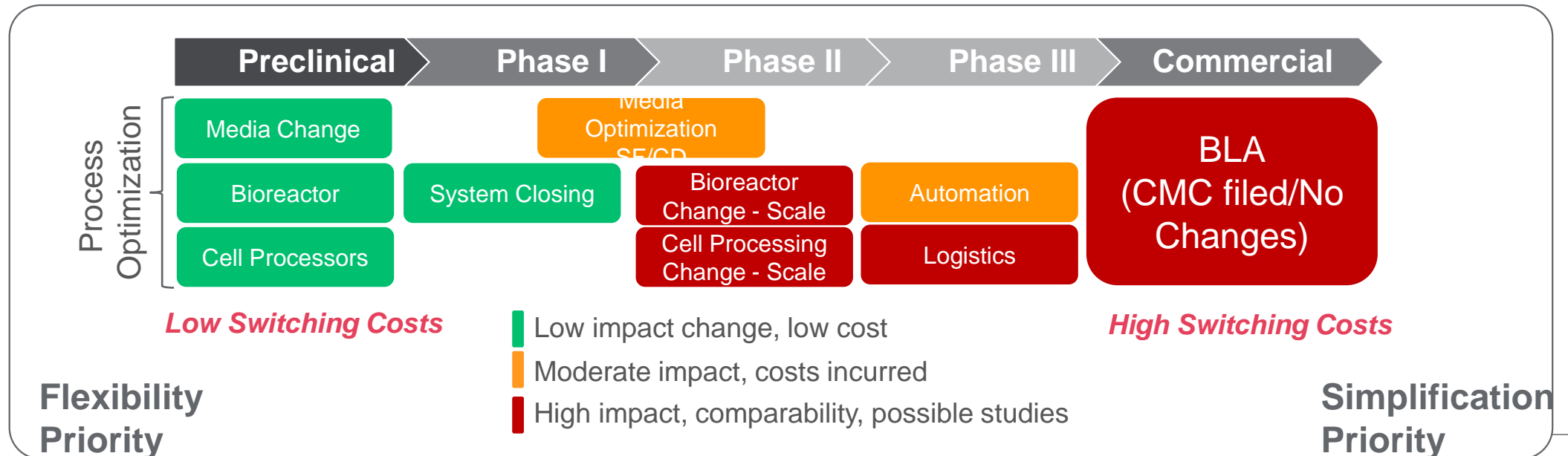
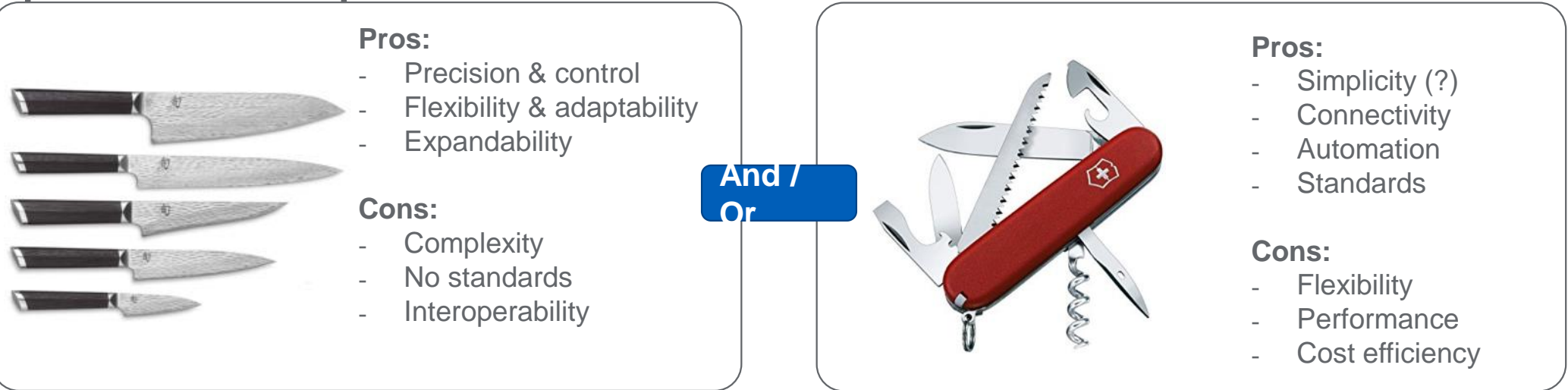
Or, find a way to use *fewer* of the “right” cells...



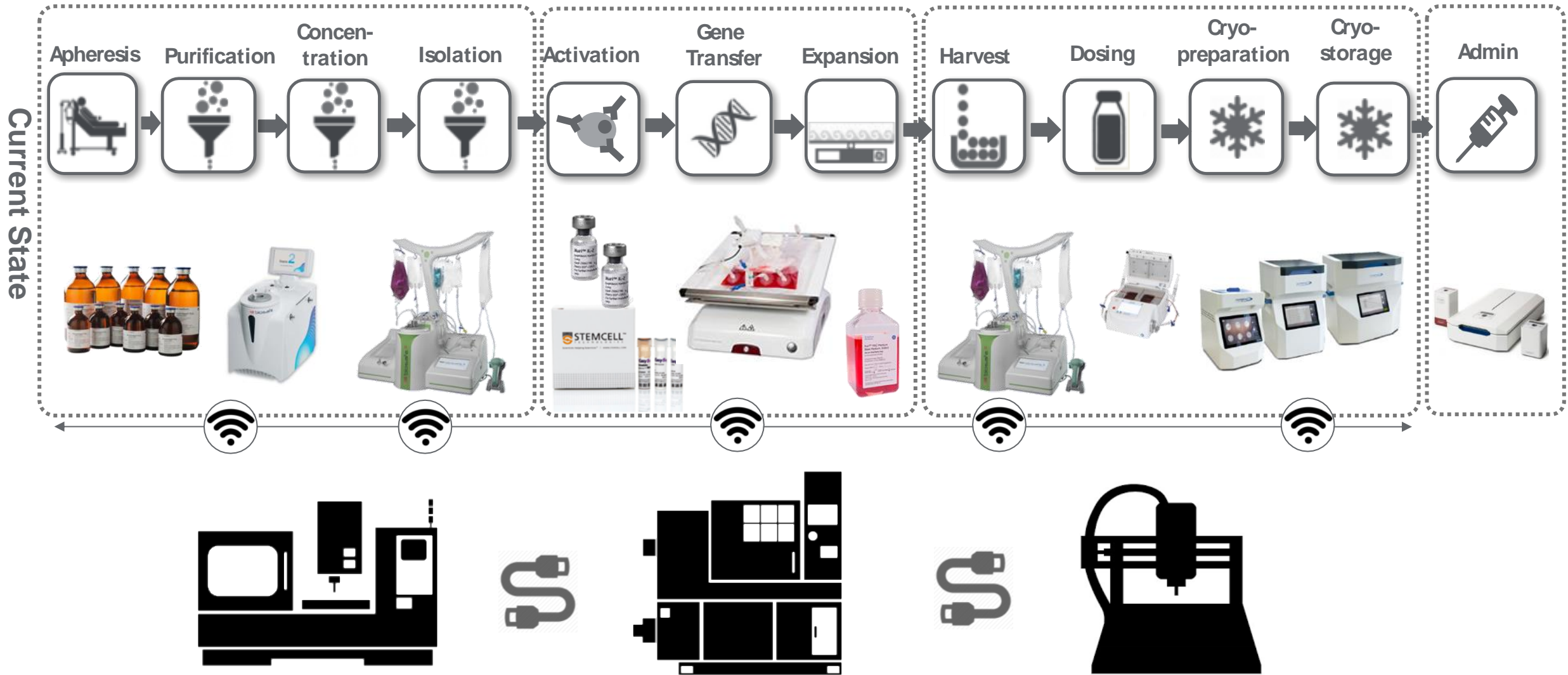
Select, enrich, or engraft



# Tool development is necessarily a compromise between bespoke unit operations and a one-size solution



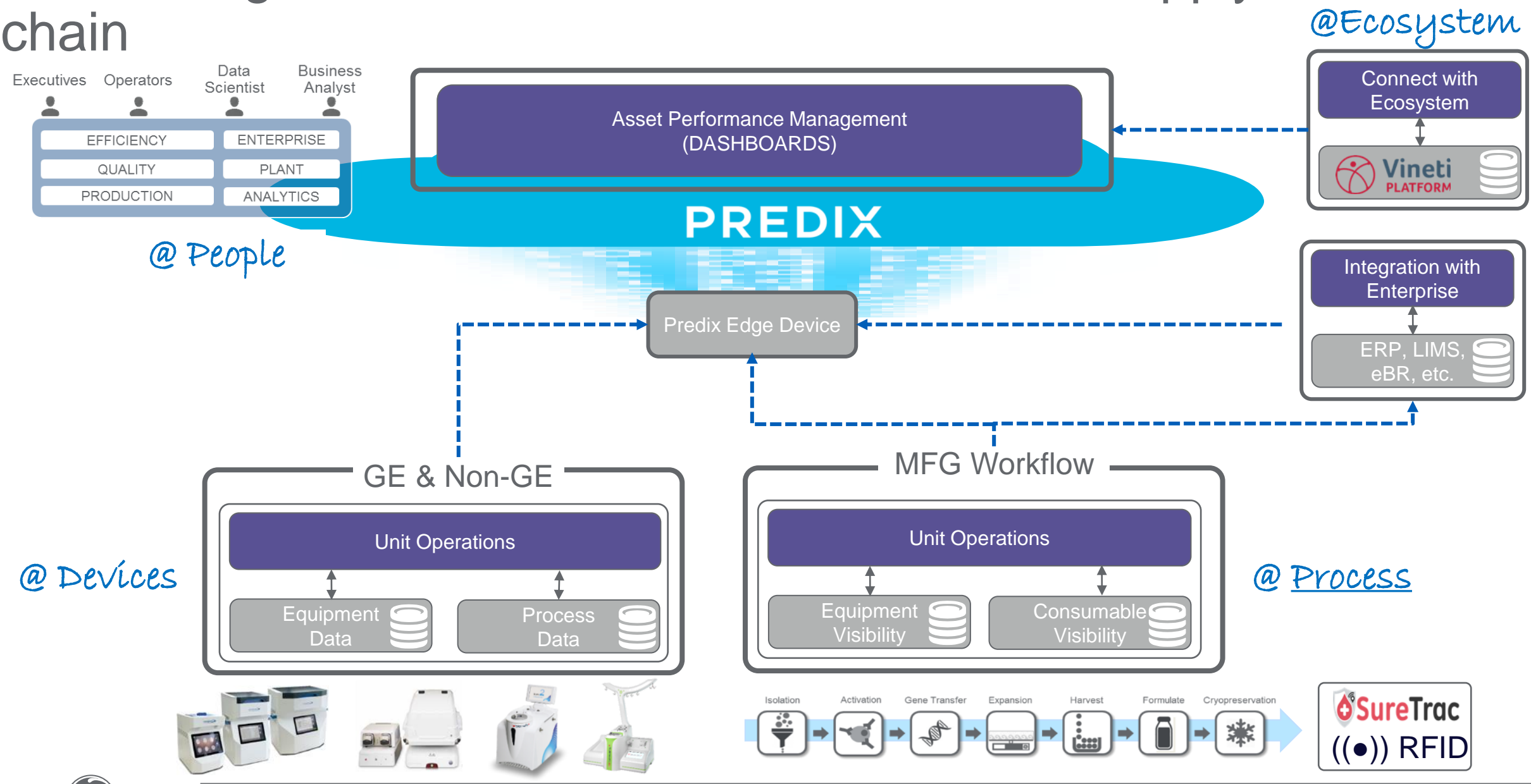
# Miniaturize, close & automate ... “Cell therapy in a box”



*Factory in a box(es) vision : 100+ step manufacturing process simplified*



# Connecting it all with a “Core to Cloud” robust supply chain





# We recognize that new, fit-for-purpose platforms & services are required to make cell therapy a widespread reality

## Key trends:

## Implications:

### *Personalization of medicine*

- Dependence on genomic / metabolomic information
- Tailoring of therapies to individuals
- Big data emergence

### *Increasing complexity of medicines / therapies*

- Increased dependence on technology
- Automation and digitization
- Deep biological understanding

### *Convergence of clinical and manufacturing pathways*

- Destination medicine
- Changes in logistics management
- Complexity of supply chain
- Medical devices



*Today, we have a sector with no clear integrator...*